

Agenda for the Public Meeting on Patient-Focused Drug Development for Fragile X Syndrome

Reminder: These discussions are based specifically on Fragile X syndrome and drug development.

| TIME | DESCRIPTION | PRESENTER/MODERATOR |
|---------------------|--|---|
| 10:00 – 10:05 AM | Welcome | Linda Sorensen Executive Director, National Fragile X Foundation |
| 10:05 – 10:10 AM | Opening Remarks | Randi Hagerman, MD University of California, Davis MIND Institute and Founder of the National Fragile X Foundation |
| 10:10 – 10:20 AM | OVERVIEW: FDA’s Patient-Focused Drug Development Initiative | Bernard Fischer, MD Deputy Director (Acting) Division of Psychiatry (DP), Office of Neuroscience (ON), Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), FDA |
| 10:20 – 10:30 AM | OVERVIEW: Fragile X Syndrome and Current Treatment Options | Elizabeth Berry-Kravis, MD, PhD Rush University |
| 10:30 – 10:40 AM | OVERVIEW: Clinical Trial Endpoints/Outcome Measures (or lack thereof) | David Hessler, PhD University of California, Davis MIND Institute |
| 10:40 – 10:45 AM | OVERVIEW: Discussion Format | Linda Sorensen Executive Director, National Fragile X Foundation |
| 10:45 – 11:15 AM | PANEL #1 DISCUSSION ON TOPIC 1* Health Effects and Daily Impacts of Fragile X Syndrome | Marcia Braden, PhD, PC Owner of a clinical private practice specializing in children and adolescents |

A panel of individuals and caregivers will provide comments to start the discussion on significant health effects and daily impacts of Fragile X syndrome.

11:15 AM – **Large-Group Facilitated Discussion** **NFXF Team**
12:15 PM **Topic 1***

Individuals and caregivers in the audience will be invited to add to the dialogue.

12:15 – **BREAK**
12:30 PM

12:30 – **PANEL #2 DISCUSSION ON TOPIC 2**** **Craig Erickson, MD**
1:00 PM Current Approaches to Treatment Cincinnati Children’s Hospital Medical
Center and Chair of the NFXF Clinical Trials
Committee

A panel of individuals and caregivers will provide comments to start the discussion on current approaches to treating Fragile X syndrome.

1:00 – **Large-Group Facilitated Discussion** **NFXF Team**
2:00 PM **Topic 2****

Individuals and caregivers in the audience will be invited to add to the dialogue.

2:00 – **Closing Remarks** **Linda Sorensen**
2:05 PM Executive Director, National Fragile X
Foundation

***Topic 1: Health Effects and Daily Impacts of Fragile X Syndrome**

1. Of all the symptoms or disease manifestations that you/your child experience(s) because of FXS, which 1-3 symptoms or manifestations have the most significant impact on your/their life?
2. Are there specific activities or skills that are important to you/your child but that you/your child cannot do at all or as fully as you/your child would like because of FXS? (Examples may include daily living or communication skills, learning or social activities)
3. How do you/your child’s symptoms and their negative impacts affect daily life on your/their best days? On the worst days?
4. How has your/your child’s FXS changed over time?
 - a. Would you define your/your child’s FXS today as being well-managed?
5. What worries you most about your/your child’s FXS?

****Topic 2: Current Approaches to Treatment**

1. What symptoms would you most like a treatment for FXS to target?
2. What are you currently doing to help treat your/your child's FXS? (Examples may include prescription medicines, over-the-counter products, and non-drug therapies such as diet modification)
 - a. How has your/your child's treatment regimen changed over time, and why?
 - b. Where do you feel are the biggest gaps in your/your child's treatment right now?
3. How well does your/your child's current treatment regimen control your/their FXS?
 - a. How well have these treatments worked as your/your child's condition has changed over time?
4. What are the most significant downsides to your/your child's current treatments, and how do they affect your/your child's daily life? (Examples of downsides may include going to the clinic for treatment, time devoted to treatment, side effects of treatment, route of administration, etc.)
5. What specific things would you look for in an ideal treatment for your/your child's FXS?
 - a. What would you consider to be a meaningful improvement in your/your child's FXS that a treatment could provide?
 - b. How big of a change/effect would you need to see to use a new treatment, assuming there will be potentially mild-moderate side effects?
 - c. What impact level would convince you to trial a treatment? (For example, a treatment that positively effects 80%, 50%, 20%, 10% of patients with FXS)
6. What factors do you take into account when making decisions about selecting a course of treatment for yourself/your child?